
A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants

Grant Award Details

A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11431

Investigator:

Name:	Judith Shizuru
Institution:	Stanford University
Type:	PI

Disease Focus: Blood Disorders, Severe Combined Immunodeficiency, X-linked (X-SCID)

Award Value: \$3,715,031

Status: Pre-Active

Grant Application Details

Application Title: A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants

Public Abstract:**Therapeutic Candidate or Device**

CD34⁺CD90⁺ hematopoietic stem cells (HSC) in combination with AMG 191, a humanized anti-CD117 monoclonal antibody

Indication

Severe Combined Immunodeficiency

Therapeutic Mechanism

AMG 191 is being utilized as a conditioning agent for selectively eliminating endogenous stem cells in pediatric SCID patients prior to CD34⁺CD90⁺ hematopoietic stem cell transplantation for repopulation of the bone marrow. Hematopoietic stem cell (HSC) transplantation possesses the ability to provide a life-long cure for all of these diverse diseases, as it allows for the replacement of defective HSC.

Unmet Medical Need

Although transplant is the proven curative treatment for SCID, this therapy has the risk of life-threatening complications and inadequate efficacy. SCID recipients are uniquely susceptible to the negative consequences of DNA-damaging chemo radiation and risk both short and long-term side effects.

Project Objective

Phase 1 trial completed

Major Proposed Activities

Complete Phase I Clinical Trial
-Enroll, treat, monitor patients
-Determine optimal dose
-Assess clinical safety and efficacy

Statement of Benefit to California:

For the health of the citizens of California, both physical and financial, we need to develop cures, rather than marginally effective treatments, for a variety of devastating blood and immune illnesses. By developing a novel, non-toxic antibody-based conditioning method, transplants could be expanded to the treatment of other diseases that are not transplanted due to associated toxicities. We hope to create definitive treatments that will lead to a reduction of the massive health care burden.

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